American Perspective

Professor Spero Cataland MD
Columbia University Medical Center

ITP and New Treatments: The view from your side of the pond

As I begin to write the latest installment of the American Perspective, I am on the way home from attending the 64th Annual ISTH Scientific and Standardization Committee (SSC) Meeting that was held in Dublin, Ireland. As always, it was an excellent meeting with expert presentations and interactions with colleagues in the field of hematology. While I had several things that I wanted to accomplish, at the top of the list was to learn more about some of the newest treatments for ITP. While I certainly learned a great deal at the meeting, the session on ITP was particularly interesting.

I thought I would be writing about fostamatinib, and new targeted therapy recently approved by the United States FDA for the treatment of chronic ITP. Fostamatinib works by blocking the destruction of antibody coated platelets, thereby leading to an increase in the platelet count. I also thought I might talk about avatrombopag, another drug recently approved drug by the FDA that could be considered very similar to romiplostim or eltrombopag. Avatrombopag works in a similar way as romiplostim or eltrombopag by boosting the bone marrow's production of platelets.

Because existing treatments already work so well for patients, I was not sure exactly where these new treatments should fit in our treatment plans for patients with ITP.

On the last day I sat in on what turned out to be an excellent and interactive session on ITP. Several experts presented lectures on different aspects of ITP, but I was particularly struck by the last lecture given by Dr. Philip Choi, a haematologist and ITP expert from Australia. In giving an excellent overview of the pathophysiology of ITP, he asked an interesting question. He asked how it was possible to really talk about the treatment of ITP and novel therapies, before we know how to diagnose ITP? On the surface this might seem like a strange question, but the point he was trying to make is that

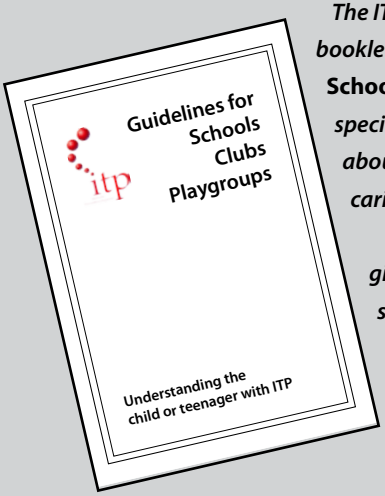
not everyone has the same "ITP". There are potential mechanisms of disease that can involve decreased production, increased destruction, with the destruction of platelets occurring in different ways. Different parts of the immune system can be involved in the autoimmune destruction of platelets in ITP including both B cells and T cells, as is evidenced by treatments that target both B and T cells being effective in the treatment of ITP. The spleen is thought to be the major site of destruction of antibody coated platelets, but not all people respond to splenectomy supporting the hypothesis that the clearing of antibody-coated platelets can occur in places other than the spleen.

Maybe in order to properly treat patients with ITP, we need to be able to "diagnose" exactly the cause of their ITP as put forth by Dr. Choi. With the addition of both fostamatinib and avatrombopag to the list of potential treatments available to physicians, we now have even more treatments that target different mechanisms of disease in ITP. By knowing which mechanism or mechanisms are causing an individual patients' ITP, we may be able to more effectively and safely treat patients with ITP that require therapy. Ongoing and future research will need to focus more on the development of testing that may be able to more accurately assess what is causing each individual's "ITP" to best know how to approach the treatment. If one looks at ITP in this new way, the potential utility of these new treatment options becomes more readily apparent.

September is the month when many children and teenagers move to new schools, toddlers start playgroups and adults enrol at gyms and sports clubs.

The ITP Support Association has two booklets to help. Our* Guidelines for Schools, Clubs and Playgroups *is specially written to explain to staff about ITP to assist them in safely caring for a youngster with ITP.

Choosing Your Sport gives advice on which sports are safe to play with ITP, and how to cope with minor injuries.



Both are free to Association members (SAE appreciated)